

ACCELERATING THE DEVELOPMENT OF DRUG CANDIDATES IN RARE DISEASES: A KEY MISSION FOR THE FRENCH FOUNDATION FOR RARE DISEASES (FFRD)

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CONTEXT

The French Foundation for rare diseases (FFRD) is a unique cooperative framework coordinating research resources and expertise in the rare diseases field. Flagship of the second French National Rare Diseases Plan, FFRD acts as a federative and strategic hub to accelerate scientific, clinical and social innovation by stimulating cross-sector cooperation towards effective healthcare to the benefit of patients affected by rare diseases. Our active support spans from basic, translational to clinical research.

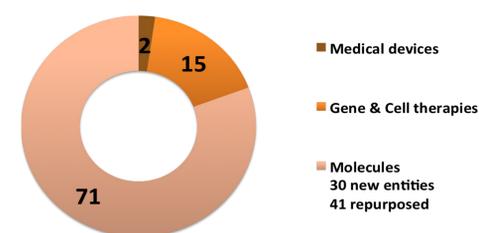
While our headquarters are located in Paris, seven regional field coordinators are in direct contact with academic and clinical teams all over the national territory to ensure that priorities are driven by grounded needs. On a daily basis, regional coordinators are dedicated to: assessing national landscape and needs in research; accelerating the translation of research into clinical development; developing rare diseases community network; detecting business development opportunities; enhancing access to innovative technologies; facilitating cross-sector partnerships. FFRD main objective is to speed up development programmes for new medicines. With its close contacts with academic researchers and clinicians in the field, FFRD is contributing to early identification of proofs of therapeutic concept and making links with other partners in the research value chain, namely the Technology Transfer Offices (TTOs).

In the same time, drug companies are turning more and more to academics to detect innovation: 17% of molecules arriving on the market are initially developed by academics (Source: Nature Reviews Drug Discovery 13, 92-93 (2014)).

THE FRENCH FOUNDATION FOR RARE DISEASES : AN INNOVATIVE MODEL TO ACCELERATE THE DEVELOPMENT OF DRUG CANDIDATES

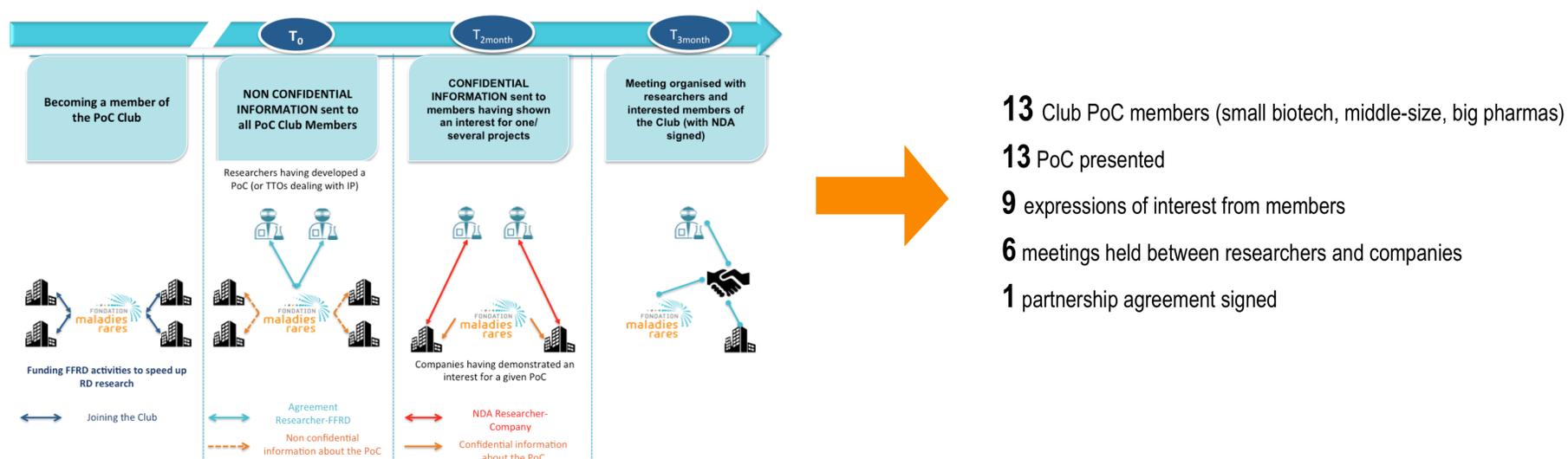
EARLY DETECTION OF THERAPEUTIC INNOVATION PROJECTS

Since 2012, 165 PoCs have been detected, 61% of them being in the pre-clinical stage. 88 of the identified PoC are actively supported by our team (sanity-check of the project - including IP and regulatory issues, identification of relevant stakeholders and partners, identification of additional funding opportunities, strategic roadmap etc.).



THE “PROMOTION OF PROOFS OF THERAPEUTIC CONCEPT” CLUB

With the wish to accelerate the development of new treatments (2% of rare diseases receive treatment today), FFRD has developed and coordinates a group of pharmaceutical and biotech companies that have an interest in the early identification and promotion of development projects for candidate medicines, the “Promotion of Proofs of Therapeutic Concept” Club. After agreement with the researchers, FFRD selects the most mature academic projects, presents them in the form of non-confidential presentations to the members of the Club. When an interest is confirmed from a member of the club, a confidentiality agreement is established between the researcher, his research organisation and TTO and the company, the latter then accessing confidential information about the project on the occasion of a meeting. The Club was launched in April 2017 and two rounds of selection of Proof of Concept Projects have already been held. This has led to a series of meetings and first partnerships have been concluded between researchers and companies confirming the role of FFRD in accelerating research valorization.



CONCLUSION

Close relationships and interactions with the various stakeholders (researchers, clinicians, TTOs, industry) and effective sourcing together with the PoC club are all success factors to accelerate the development of drug candidates in rare diseases: a key mission for the French Foundation for rare diseases.